

List of interventional PNH clinical trials marked "recruiting" or "not yet recruiting" as of January 5th 2024

company	ADARx	Apellis	BioCryst	BioCryst	Care Pharma	Fred Hutchinson Can Kira Pharmaceuticals	NHLBI	NHLBI	Novartis	Novartis	NovelMed	NovelMed	NovelMed	Omeros	Omeros	Regeneron	Regeneron	Roche	Wuhan Create	Wuhan Create	
trial	ADX-038-101	APL2-PNH-209	BCX930-205 2021-006776-17	BCX10013-105	CAN 106-PNH-102/201	BMTCTN1904 KP104-201	100154 0154	10-H-180090 18-h-0090	CLNPQ23C12001B CLNPQ23C12303	NM8074	NM8074	NM8074	NM8074-PNH-101	OMS906-PNH-002	OMS906-PNH-001	R3918-PNH-2021 ACCESS-1	2050-2021-004931-10	BO24161 COMMODORE 1	MY008211A-PNH-2-01	MY008211A-PNH-2-01-F	
ClinicalTrials ID	NCT05876312	NCT04901936	NCT05741346	NCT06100900	NCT05539248	NCT04965597 NCT05476887	NCT01174108	NCT03520647	NCT04747613	NCT05630001	NCT05646524	NCT05646563	NCT05731050	NCT05889299	NCT05972967	NCT05133531	NCT05744921	NCT04432584	NCT06050226	NCT06134414	
name	ADX-038	APL-2 Pegcetacoplan Empaveli®	BCX9930	BCX10013	CAN106	treosulfan, fludarabine and rATG		Cyclophosphamide	LNP023 Iptacopan	Iptacopan			NM8074	OMS906	OMS906		Pozelimab and Cemdisiran	Pozelimab and Cemdisiran	Crovalimab	MY008211A	MY008211A
trade name																					
determination	genes		Factor D	Factor D	CS	BMT	alt. + term. pathway	BMT	BMT	Fabhalta® Factor B	Fabhalta® Factor B	anti-Factor Bb	anti-Factor Bb	anti-Factor Bb	MASP-3	MASP-3	CS	CS	CS	hemolysis	hemolysis
substance	sRNA duplex oligonucleotide	Cyclopeptide			monoclonal antibody	cytostatics, cytotoxins and antibodies	biologic	allogenic stem cell therapy	cytostatic	low molecular compound	low molecular compound	monoclonal antibody	monoclonal antibody				monoclonal antibody I.v./then s.c.	monoclonal antibody and RNAi	monoclonal antibody		
application	s.c.	oral	oral	oral	I.v.	I.v.	I.v.	I.v.	oral	oral		I.v.	I.v.	s.c.	I.v.	I.v.	s.c.	s.c.	single I.v./then s.c.	oral	oral
frequency		2 x weekly	2xdaily	daily for 4 weeks, then maybe escalation	every 4 or every 8 weeks	6 days daily until transplantation, following 180 days of prophylaxis	weekly/biweekly	single infusion combined with various medicaments	once, hospital stay over weeks	2 x daily	2 x daily	every 2 weeks	every 4 weeks/2 weeks	every two weeks, total of 6 doses	every four weeks 1b	every eight weeks		every four weeks	every 4 weeks	2x daily	2 x daily
phase of study	1	2	2	1	2	2	2	2	3	3	2	2	2	2	1b	2 3		3 3		2	2
research question	safety, tolerability, pk and pd of adx-038 in healthy volunteers and pnh- patients	pediatric recruiting	continued treatment for patients already receiving this drug	evaluation of safety, tolerability, pharmacokinetics, pharmacodynamics and therapeutic potential of BCX10013	assessment of safety, tolerability, efficacy, pc and pc in complement- inhibitor treatment- naive and pretreated adults	inhibitor-naive patients, dose and efficacy assessment	variation of allogenic stem cell transplantation	haplo-identical transplantation and post-transplant cyclophosphamide in sAA and PNH	long-term evaluation	patients on stable eculizumab or ravulizumab treatment and Hb<10 g/dl	treatment-naive patients, assessment of safety, efficacy and addon therapy to immunosuppression in 2 dosing schemes	mono vs eculizumab	safety and efficacy of NM8074 in Sotiris-treated pnh patients	recruiting	recruiting	safety, tolerability,pharmok inetics, pharmacodynamics & efficacy when ravulizumab has failed	versus ravulizumab, not recently inhibitor treated	safety, efficacy and tolerability of combination therapy	efficacy of MY008211A in patients with signs of acute hemolysis	efficacy of MY008211A in patients with signs of acute hemolysis	
status	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	not yet recruiting	not yet recruiting	not yet recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	recruiting	not yet recruiting
start	07/2023	02/2021	01/2023	10/2023	03/2022	04/2022	12/2010	02/2019	07/2021	04/2023	11/2023	9/2023	06/2025	12/2022	03/2023	8/2022	03/2023	09/2020	07/2023	12/2024	
locations	Australia	France Malaysia Netherlands Serbia Spain Thailand UK USA	France Hungary Korea Malaysia Spain UK	South Africa	China	USA	USA	USA	Brazil China Czechia France Germany Italy Japan Lithuania Malaysia Netherlands Singapore South Korea Spain Taiwan UK USA	tdb	tdb	tdb	Ukraine	Germany Greece Switzerland UK	Singapore South Korea Taiwan USA	Germany Greece South Korea Taiwan USA	Korea	Australia Belgium Brazil Canada Czechia Estonia France Germany Greece Hong Kong Hungary Ireland Italy Japan Netherlands Poland Portugal Saudi Arabia Singapore South Korea Spain Sweden Taiwan Turkey UK USA	China	China	
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked recruiting or not yet recruiting.																				
Source	ClinicalTrials.gov (https://clinicaltrials.gov/ct2/home)																				
Last updated	05 January 2024																				

List of interventional PNH clinical trials marked "active, not recruiting" or "enrolling by invitation" as of January 5th 2024

company	Akari	Alexion	Alexion	Alexion	Alexion	Apellis	BioCryst	BioCryst	BioCryst	City of Hope Medical	Medicine Co.	Roche	Roche	Roche	Roswell
trial	AK585		ALXN2040-PNH-301					BCX9930-203	BCX9930-203	1089		BP39144	BO42162	YO42311	I40916
ClinicalTrials ID	CONSENT II NCT03427060	ACH228-110 NCT04170023	ALPHA NCT04469465	ALXN2040-PNH-303 NCT05389449	D7414C00001 NCT05886244	APL2-307 NCT0531255	BCX9930-201 NCT04702568	REDEEM-1 NCT05116774	REDEEM-2 NCT05116787	P30CA033572 NCT00544115	HRS-5965-202 NCT06051357	COMPOSER NCT03157635	COMMODORE 2 NCT04434092	COMMODORE 3 NCT04654468	NCI-2017-01949 NCT03333486
name	Nomacopan/ Coversin	ALXN2050 (ACH-0145) Danicopan	ALXN2040 Danicopan	ALXN2040, ACH-0144471, Danicopan	Eculizumab	APL-2 Pegcetacoplan Empaveli* Aspaveli*	BCX9930	BCX9930	BCX9930	stem cells from donor	HRS-5965	Crovalimab	Crovalimab	Crovalimab	
trade name			Voydeya*	Voydeya*	Soliris*										
determination	CS	Factor D	low molecular compound	low molecular compound	monoclonal antibody	Cyclopeptide	low molecular compound	low molecular compound	low molecular compound	stem cells		CS	CS	CS	monoclonal antibody
substance application	tick saliva protein s.c.	oral	oral	oral	i.v. once a week for 22 days, followed by once every two weeks	s.c.	oral	oral	oral		oral	monoclonal antibody i.v. / s.c.	monoclonal antibody single i.v./then s.c.	monoclonal antibody single i.v./then s.c.	i.v.
frequency	2 x daily		3 x daily	3 x daily		2 x weekly	2 x daily	2 x daily	2 x daily			every 4 weeks	every 4 weeks	every 4 weeks	at least 6 days
phase of study	2	2	3	3	3	3	2	2	2	2	2	1/2	3	3	2
research question	patients with resistance to eculizumab	safety and efficacy of ALXN2050 as a monotherapy	add-on therapy to ravulizumab, ALXN2040 vs placebo	LTE add on to C5i treatment in patients previously treated with danicopan	safety of eculizumab	long-term evaluation	long-term evaluation	monotherapy vs CS inhibitor in pre-treated patients who are still anemic	monotherapy vs placebo in treatment-naive patients	donor peripheral stem cell transplant in patients with hematologic disorders	efficacy, safety of HRS-5965		versus eculizumab, treatment-naive	treatment naive	combination of different therapies to treat blood cancer
status	enrolling by invitation	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting	active, not recruiting
start	05/2018	12/2019	12/2020	10/2022	07/2023	08/2018	12/2020	12/2021	10/2021	10/2001	11/2023	11/2016	10/2020	03/2021	12/2017
locations	USA	Canada Italy Korea New Zealand Spain Turkey UK	Brazil Canada Czechia France Germany Greece Israel Italy Japan Malaysia Netherlands Poland South Korea Spain Taiwan Thailand UK USA	UK USA Italy	China	Australia Belgium Bulgaria Canada France Germany Hong Kong Japan Malaysia Russia Serbia Singapore South Korea Spain Thailand UK USA	Austria South Africa UK	France Hungary Italy Spain UK	Malaysia South Africa	tbd	China	France Germany Hungary Italy Japan Netherlands South Korea	Argentina Australia Brazil China France Germany Greece Hong Kong Japan Lithuania Malaysia Mexico Netherlands Philippines Poland Portugal Romania Singapore South Africa	China	USA
Remarks	This table contains clinical trials listed under ClinicalTrials.gov that are marked active/not recruiting or enrolling by invitation and have no results published.														
Source	ClinicalTrials.gov (https://clinicaltrials.gov/ct2/home)														
Last updated	05 January 2024														